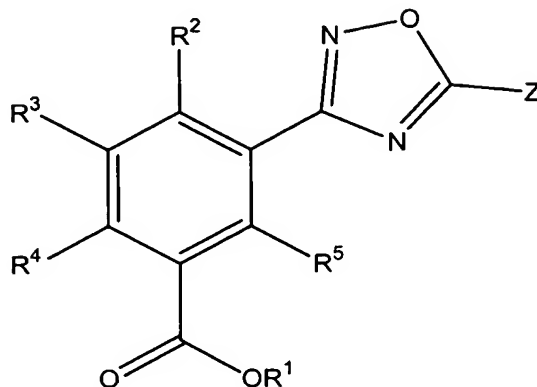


What is claimed is:

1. A compound of the formula:



wherein:

Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl;

R<sup>1</sup> is hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, -(CH<sub>2</sub>CH<sub>2</sub>)<sub>n</sub>OR<sup>6</sup> or any biohydrolyzable group;

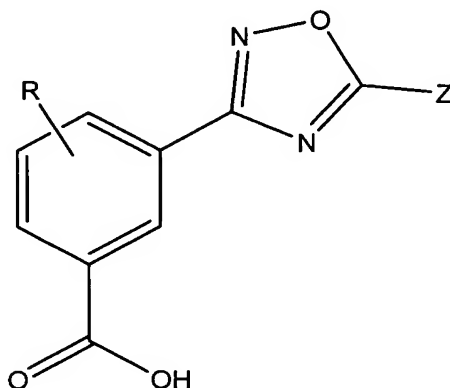
R<sup>2</sup>, R<sup>3</sup>, R<sup>4</sup>, R<sup>5</sup> and R<sup>6</sup> are independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen, CF<sub>3</sub>, OCF<sub>3</sub>, OCHF<sub>2</sub>, CN, COOH, COOR<sup>7</sup>, SO<sub>2</sub>R<sup>7</sup>, NO<sub>2</sub>, NH<sub>2</sub>, or N(R<sup>7</sup>)<sub>2</sub>;

each occurrence of R<sup>7</sup> is independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen or CF<sub>3</sub>;

n is an integer from 1 to 7;

with the proviso that when  $R^1$ ,  $R^2$ ,  $R^3$ ,  $R^4$ , and  $R^5$  are each hydrogen, Z is not methyl, 2-carboxy ethyl, 3-(4-pyridinyl)propyl, or 2-(4-piperidinyl) ethyl.

2. The compound of claim 1 having the formula II:



or pharmaceutically acceptable salts, hydrates, clathrates, or stereoisomers thereof wherein Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl; and R is hydrogen or halogen.

3. The compound of claim 1 wherein  $R^1$  is a biohydrolyzable ester.

4. The compound of claim 1 or 2 wherein Z is p-Tolyl; (4-Chloromethyl-phenyl); (2-Chloro-pyridin-3-yl); (2-Fluoro-phenyl); (3,4-Difluoro-phenyl); (4-Methoxy-phenyl); Benzo[1,3]dioxol-yl; (4-Ethyl-phenyl); o-Tolyl; (2-Chloro-phenyl); (3-Methyl-thiophen-2-yl); Benzo[b]thiophen-2-yl; (3-Fluoro-phenyl); (4-tert-Butyl-phenyl); (2-Methoxy-phenyl); (2,Difluoro-phenyl); Thiophen-2-yl; (2,4-Difluoro-phenyl); (3-Chloro-phenyl); m-Tolyl; (4-Trifluoromethyl-phenyl); (4-Fluoro-phenyl); (3-Methoxy-phenyl); Phenyl; (2,6-Difluoro-phenyl); (2,Dimethyl-furan-3-yl); (4-Pyrrol-1-yl-phenyl); (3-Dimethylamino-phenyl); Biphenyl-4-yl; (4-Dimethylamino-phenyl); Benzo[1,2,5]oxadiazol-yl; m-Tolyl; (2-Trifluoromethyl-phenyl); (6-Chloro-pyridin-3-yl); (3,Bis-trifluoromethyl-phenyl); Furan-2-yl; (4-Nitro-phenyl); (3,4-Dimethoxy-phenyl); (3-Trifluoromethoxy-phenyl); Naphthalen-1-yl; Cyclohexyl; Pyridin-3-yl; Pyridin-4-yl; Cyclopentyl; Cyclopropyl; (4-Pentyloxy-phenyl); (3,4,Trimethoxy-phenyl); (4-Isobutyl-phenyl); Cyclobutyl; (1-Acetyl-piperidin-4-yl); Isoxazol-yl; [2-Chloro-6-fluoro-phenyl]-methyl-isoxazol-4-yl or [2-Chloro-phenyl]-methyl-isoxazol-4-yl].

5. A compound selected from the group consisting of:

3-(5-p-Tolyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(4-Chloromethyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(2-Chloro-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(3,4-Difluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(4-Methoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Benzo[1,3]dioxol-5-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(4-Ethyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-o-Tolyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(2-Chloro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(3-Methyl-thiophen-2-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Benzo[b]thiophen-2-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(3-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(4-tert-Butyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(2-Methoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(2,5-Difluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Thiophen-2-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(2,4-Difluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(3-Chloro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-m-Tolyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(4-Trifluoromethyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(4-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(3-Methoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Phenyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(2,6-Difluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(2,5-Dimethyl-furan-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(4-Pyrrol-1-yl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(3-Dimethylamino-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Biphenyl-4-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(4-Dimethylamino-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-(5-Benzo[1,2,5]oxadiazol-5-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-(5-m-Tolyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

3-[5-(2-Trifluoromethyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(6-Chloro-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3,5-Bis-trifluoromethyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Furan-2-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-[5-(4-Nitro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3,4-Dimethoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3-Trifluoromethoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Naphthalen-1-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Cyclohexyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Pyridin-3-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Pyridin-4-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Cyclopentyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Cyclopropyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-[5-(4-Pentyloxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3,4,5-Trimethoxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(4-Isobutyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Cyclobutyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-[5-(1-Acetyl-piperidin-4-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Isoxazol-5-yl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-{5-[3-(2-Chloro-6-fluoro-phenyl)-5-methyl-isoxazol-4-yl]-[1,2,4]oxadiazol-3-yl}-  
 benzoic acid;  
 3-(5-Isopropyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-tert-Butyl-[1,2,4]oxadiazol-3-yl)-benzoic acid; 3-(5-Butyl-[1,2,4]oxadiazol-3-  
 yl)-benzoic acid;  
 3-(5-Propenyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-[5-(4-Chloro-benzyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(4-Chloro-phenoxy-methyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Benzyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-(5-Methoxymethyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;  
 3-[5-(1-Phenyl-propyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(4-Fluoro-benzyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3-Chloro-phenoxy-methyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(6-Chloro-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-(5-Cyclopentylmethyl-[1,2,4]oxadiazol-3-yl)-benzoic acid;

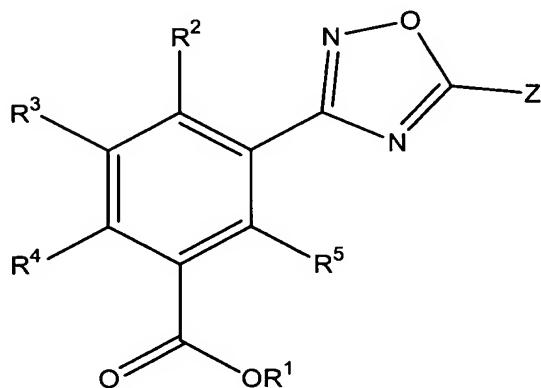
3-[5-(4-Methoxy-benzyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2,3-Difluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2-Fluoro-5-methyl-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2-Methylsulfanyl-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2,2-Difluoro-benzo[1,3]dioxol-5-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 4-Fluoro-3-[5-(4-fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 2-Fluoro-5-[5-(4-fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(4-Chloro-2-fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(4-Bromo-2-fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3-Fluoro-biphenyl-4-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-{5-[3-(2-Chloro-phenyl)-5-methyl-isoxazol-4-yl]-[1,2,4]oxadiazol-3-yl}-benzoic acid;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid sodium salt;  
 3-[5-(4-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid methyl ester;  
 5-[5-(4-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-2-methoxy-benzoic acid;  
 3-[5-(3-Fluoro-biphenyl-4-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(6-Pyrrolidin-1-yl-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(6-Morpholin-4-yl-pyridin-3-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(3,4,5,6-Tetrahydro-2H-[1,2']bipyridinyl-5'-yl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2-Fluoro-6-hydroxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid methyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-methoxy-ethyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-(2-methoxy-ethoxy)-ethyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-[2-(2-methoxy-ethoxy)-ethoxy]-ethyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-{2-[2-(2-methoxy-ethoxy)-ethoxy]-ethoxy}-ethyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-(2-{2-[2-(2-methoxy-ethoxy)-ethoxy]-ethoxy}-ethoxy)-ethyl ester;  
 3-[5-(2-Fluoro-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid 2-[2-(2-{2-[2-(2-hydroxy-ethoxy)-ethoxy]-ethoxy}-ethoxy)-ethoxy]-ethyl ester;  
 3-[5-(4-Amino-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid;

3-[5-(4-Azido-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid; and  
 3-[5-(4-Benzyloxy-phenyl)-[1,2,4]oxadiazol-3-yl]-benzoic acid  
 and pharmaceutically acceptable salts, hydrates, solvates, clathrates and  
 stereoisomers thereof.

6. A pharmaceutical composition comprising a compound according to claim 1  
 or 5 and a pharmaceutically acceptable carrier.

7. A unit dosage form comprising a compound according to claim 1 or 5 and a  
 pharmaceutically acceptable carrier.

8. A method for modulating premature translation termination or nonsense-  
 mediated mRNA decay in a cell comprising contacting a cell exhibiting premature  
 translation termination or nonsense-mediated mRNA decay with an effective amount of a  
 compound of the formula:



wherein:

Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl,  
 substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl,  
 substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle,  
 substituted or unsubstituted arylalkyl;

R<sup>1</sup> is hydrogen, substituted or unsubstituted alkyl, substituted or  
 unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted  
 or unsubstituted aryl, substituted or unsubstituted heteroaryl, -(CH<sub>2</sub>CH<sub>2</sub>)<sub>n</sub>OR<sup>6</sup> or any  
 biohydrolyzable group;

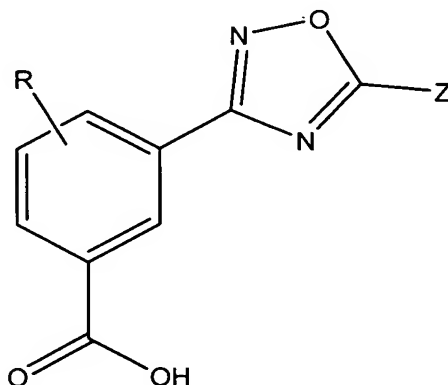
R<sup>2</sup>, R<sup>3</sup>, R<sup>4</sup>, R<sup>5</sup> and R<sup>6</sup> are independently hydrogen, substituted or  
 unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted

alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen, CF<sub>3</sub>, OCF<sub>3</sub>, OCHF<sub>2</sub>, CN, COOH, COOR<sup>7</sup>, SO<sub>2</sub>R<sup>7</sup>, NO<sub>2</sub>, NH<sub>2</sub>, or N(R<sup>7</sup>)<sub>2</sub>;

each occurrence of R<sup>7</sup> is independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen or CF<sub>3</sub>; and

n is an integer from 1 to 7.

9. The method of claim 8 comprising contacting a cell exhibiting premature translation termination or nonsense-mediated mRNA decay with an effective amount of a compound of the formula **II**:



or pharmaceutically acceptable salts, hydrates, clathrates, or stereoisomers thereof wherein Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl; and R is hydrogen or halogen.

10. The method of claim 8 or 9 wherein said disease is a genetic disease.

11. The method of claim 8 or 9 wherein said genetic disease is an autoimmune disease, a blood disease, a collagen disease, diabetes, an inflammatory disease, or a central nervous system disease.

12. The method of claim 11, wherein the autoimmune disease is rheumatoid arthritis or graft versus host disease.

13. The method of claim 11, wherein the inflammatory disease is arthritis.

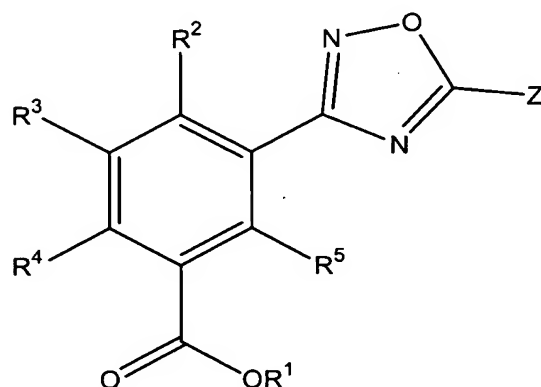
14. The method of claim 11, wherein the central nervous system disease is multiple sclerosis, muscular dystrophy, late infantile neuronal ceroid lipofuscinosis, Duchenne muscular dystrophy, Alzheimer's disease, Tay Sachs disease, a neurodegenerative disease or Parkinson's disease.

15. The method of claim 11, wherein the blood disease is hemophilia, Von Willebrand disease, ataxia-telangiectasia, b-thalassemia or kidney stones.

16. The method of claim 11, wherein the collagen disease is osteogenesis imperfecta or cirrhosis.

17. The method of claim 10, wherein the genetic disease is familial polycythemia, immunodeficiency, kidney disease, cystic fibrosis, familial hypercholesterolemia, retinitis pigmentosa, amyloidosis, atherosclerosis, gigantism, dwarfism, hypothyroidism, hyperthyroidism, aging, obesity, Niemann Pick's disease or Marfan syndrome.

18. A method of treating or preventing a genetic disease, or ameliorating one or more symptoms associated with or manifestations of a genetic disease comprising administering to a patient in need thereof a therapeutically or prophylactically effective amount of a compound of the formula:





wherein:

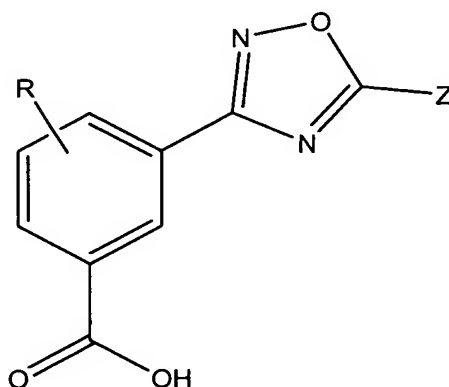
Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl;

R<sup>1</sup> is hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, -O(CH<sub>2</sub>CH<sub>2</sub>)<sub>n</sub>OR<sup>6</sup> or any biohydrolyzable group;

R<sup>2</sup>, R<sup>3</sup>, R<sup>4</sup>, R<sup>5</sup> and R<sup>6</sup> are independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen, CF<sub>3</sub>, OCF<sub>3</sub>, OCHF<sub>2</sub>, CN, COOH, COOR<sup>7</sup>, SO<sub>2</sub>R<sup>7</sup>, NO<sub>2</sub>, NH<sub>2</sub>, or N(R<sup>7</sup>)<sub>2</sub>; each occurrence of R<sup>7</sup> is independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen or CF<sub>3</sub>; and

n is an integer from 1 to 7.

19. The method of claim 18 comprising administering to a patient in need thereof a therapeutically or prophylactically effective amount of a compound of the formula **II**:



or pharmaceutically acceptable salts, hydrates, clathrates, or stereoisomers thereof wherein Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl; and R is hydrogen or halogen.

20. The method of claim 18 or 19 wherein said genetic disease is an autoimmune disease, a blood disease, a collagen disease, diabetes, an inflammatory disease, or a central nervous system disease.

21. The method of claim 20, wherein the autoimmune disease is rheumatoid arthritis or graft versus host disease.

22. The method of claim 20, wherein the inflammatory disease is arthritis.

23. The method of claim 20, wherein the central nervous system disease is multiple sclerosis, muscular dystrophy, late infantile neuronal ceroid lipofuscinosis, Duchenne muscular dystrophy, Alzheimer's disease, Tay Sachs disease, a neurodegenerative disease or Parkinson's disease.

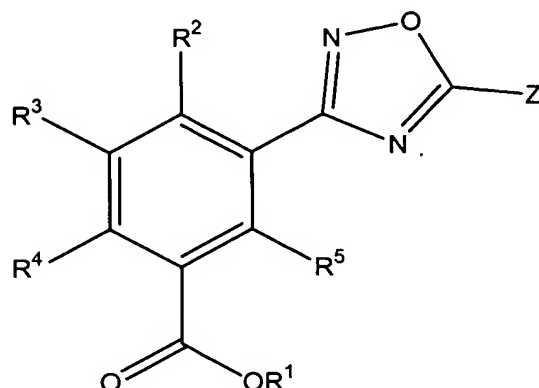
24. The method of claim 20, wherein the blood disease is hemophilia, Von Willebrand disease, ataxia-telangiectasia, b-thalassemia or kidney stones.

25. The method of claim 20, wherein the collagen disease is osteogenesis imperfecta or cirrhosis.

26. The method of claim 8 or 9, wherein the genetic disease is familial polycythemia, immunodeficiency, kidney disease, cystic fibrosis, familial hypercholesterolemia, retinitis pigmentosa, amyloidosis, atherosclerosis, gigantism, dwarfism, hypothyroidism, hyperthyroidism, aging, obesity, Neimann Pick's disease, or Marfan syndrome.

27. A method of treating, preventing or ameliorating cancer or one or more symptoms associated with or manifestations of cancer comprising administering to a patient

in need thereof a therapeutically or prophylactically effective amount of a compound of the formula:



wherein:

Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl;

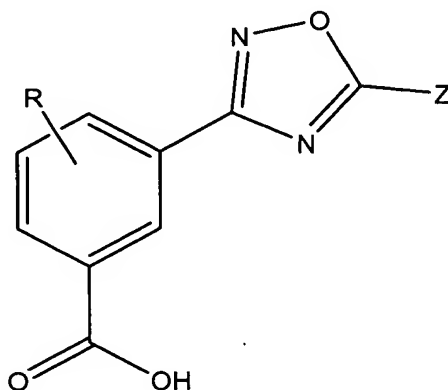
R¹ is hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl,  $-(CH_2CH_2)_nOR^6$  or any biohydrolyzable group;

R², R³, R⁴, R⁵ and R⁶ are independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen, CF₃, OCF₃, OCHF₂, CN, COOH, COOR⁷, SO₂R⁷, NO₂, NH₂, or N(R⁷)₂;

each occurrence of R⁷ is independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen or CF₃; and

n is an integer from 1 to 7.

28. The method of claim 27 comprising administering to a patient in need thereof a therapeutically or prophylactically effective amount of a compound of the formula II:



or pharmaceutically acceptable salts, hydrates, clathrates, or stereoisomers thereof wherein Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl; and R is hydrogen or halogen.

29. The method of claim 27 or 28 wherein said cancer is of the head and neck, eye, skin, mouth, throat, esophagus, chest, bone, lung, colon, sigmoid, rectum, stomach, prostate, breast, ovaries, kidney, liver, pancreas, brain, intestine, heart, adrenals, a solid tumor, sarcoma, carcinomas, fibrosarcoma, myxosarcoma, liposarcoma, chondrosarcoma, osteogenic sarcoma, chordoma, angiosarcoma, endotheliosarcoma, lymphangiosarcoma, lymphangioendotheliosarcoma, synovioma, mesothelioma, Ewing's tumor, leiomyosarcoma, rhabdomyosarcoma, squamous cell carcinoma, basal cell carcinoma, adenocarcinoma, sweat gland carcinoma, sebaceous gland carcinoma, papillary carcinoma, papillary adenocarcinomas, cystadenocarcinoma, medullary carcinoma, bronchogenic carcinoma, renal cell carcinoma, hepatoma, bile duct carcinoma, choriocarcinoma, seminoma, embryonal carcinoma, Wilms' tumor, cervical cancer, testicular tumor, lung carcinoma, small cell lung carcinoma, bladder carcinoma, epithelial carcinoma, glioma, astrocytoma, medulloblastoma, craniopharyngioma, ependymoma, Kaposi's sarcoma, pinealoma, hemangioblastoma, acoustic neuroma, oligodendroglioma, meningioma, melanoma, neuroblastoma, retinoblastoma, a blood-born tumor, acute lymphoblastic leukemia, acute lymphoblastic B-cell leukemia, acute lymphoblastic T-cell leukemia, acute myeloblastic leukemia, acute promyelocytic leukemia, acute monoblastic leukemia, acute

erythroleukemic leukemia, acute megakaryoblastic leukemia, acute myelomonocytic leukemia, acute nonlymphocytic leukemia, acute undifferentiated leukemia, chronic myelocytic leukemia, chronic lymphocytic leukemia, hairy cell leukemia, multiple myeloma, or p53-associated.

30. The method of claim 18, 19, 27 or 28 wherein the patient is a mammal.

31. The method of claim 18, 19, 27 or 28 wherein the compound is administered parenterally, transdermally, mucosally, nasally, buccally, sublingually, or orally.

32. The method of claim 31 wherein the compound is administered orally.

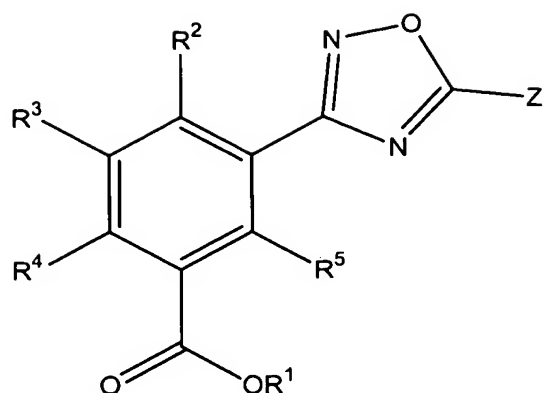
33. The method of claim 32 wherein the compound is administered orally in a tablet, liquid or capsule form.

34. The method of claim 18, 19, 27 or 28 wherein the therapeutically or prophylactically effective amount is from about 1 mg to about 2000 mg per day.

35. The method of claim 34 wherein the therapeutically or prophylactically effective amount is from about 5 mg to about 500 mg per day.

36. The method of claim 35 wherein the therapeutically or prophylactically effective amount is from about 10 mg to about 200 mg per day.

37. A method of treating, preventing or ameliorating one or more symptoms associated with or manifestations of an autoimmune disease, a blood disease, a collagen disease, diabetes, an inflammatory disease, or a central nervous system disease comprising administering to a patient in need thereof an effective amount of a compound of the formula:



wherein:

Z is substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted heterocycle, substituted or unsubstituted arylalkyl;

R<sup>1</sup> is hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl,  $-(CH_2CH_2)_nOR^6$  or any biohydrolyzable group;

R<sup>2</sup>, R<sup>3</sup>, R<sup>4</sup>, R<sup>5</sup> and R<sup>6</sup> are independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen, CF<sub>3</sub>, OCF<sub>3</sub>, OCHF<sub>2</sub>, CN, COOH, COOR<sup>7</sup>, SO<sub>2</sub>R<sup>7</sup>, NO<sub>2</sub>, NH<sub>2</sub>, or N(R<sup>7</sup>)<sub>2</sub>;

each occurrence of R<sup>7</sup> is independently hydrogen, substituted or unsubstituted alkyl, substituted or unsubstituted alkenyl, substituted or unsubstituted alkynyl; substituted or unsubstituted cycloalkyl, substituted or unsubstituted heterocycloalkyl, substituted or unsubstituted aryl, substituted or unsubstituted heteroaryl, alkoxy, aryloxy, heteroaryloxy, halogen or CF<sub>3</sub>; and

n is an integer from 1 to 7..

38. The method of claim 37 wherein said disease is rheumatoid arthritis, graft versus host disease, arthritis, multiple sclerosis, muscular dystrophy, late infantile neuronal ceroid lipofuscinosis, Duchenne muscular dystrophy, Alzheimer's disease, Tay Sachs disease, a neurodegenerative disease, Parkinson's disease, Niemann Pick's disease,

hemophilia, Von Willebrand disease, ataxia-telangiectasia, b-thalassemia, kidney stones, osteogenesis imperfecta, cirrhosis, familial polycythemia, immunodeficiency, kidney disease, cystic fibrosis, familial hypercholesterolemia, retinitis pigmentosa, amyloidosis, atherosclerosis, gigantism, dwarfism, hypothyroidism, hyperthyroidism, aging, obesity, or Marfan syndrome.